DMD CARE UK

Best care for all

DMD Care UK Annual Meeting Report 2025

Newcastle University

NHS The Newcastle upon Tyne Hospitals NHS Foundation Trust

THE John Walton MUSCULAR DYSTROPHY RESEARCH CENTRE

Duchenne UK

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Background

ABOUT DMD CARE UK

DMD Care UK is a nationwide initiative established by the John Walton Muscular Dystrophy Research Centre at Newcastle University, and Duchenne UK. The project is embedded in the North Star Clinical Network with the core goal of improving and harmonising clinical care for everyone living with Duchenne muscular dystrophy (DMD) in the UK.

We achieve this by taking the principles presented in the 2018 international care guidelines (Birnkrant et al., 2018, Lancet Neurology) and work with leading multidisciplinary professionals in clinical practice as well as patient experts to reach consensus on practical guidelines that are relevant to UK clinical practice.

DMD Care UK are currently working with these organisations to increase the impact of our care guidelines:

- British Cardiovascular Society
- British Thoracic Society
- British Society for Paediatric Endocrinology and Diabetes
- Chartered Society of Physiotherapy
- British Society for Children's Orthopaedic Surgery
- British Society of Echocardiography
- British Society of Cardiovascular Magnetic Resonance
- National Institute for Health and Care Excellence (NICE)



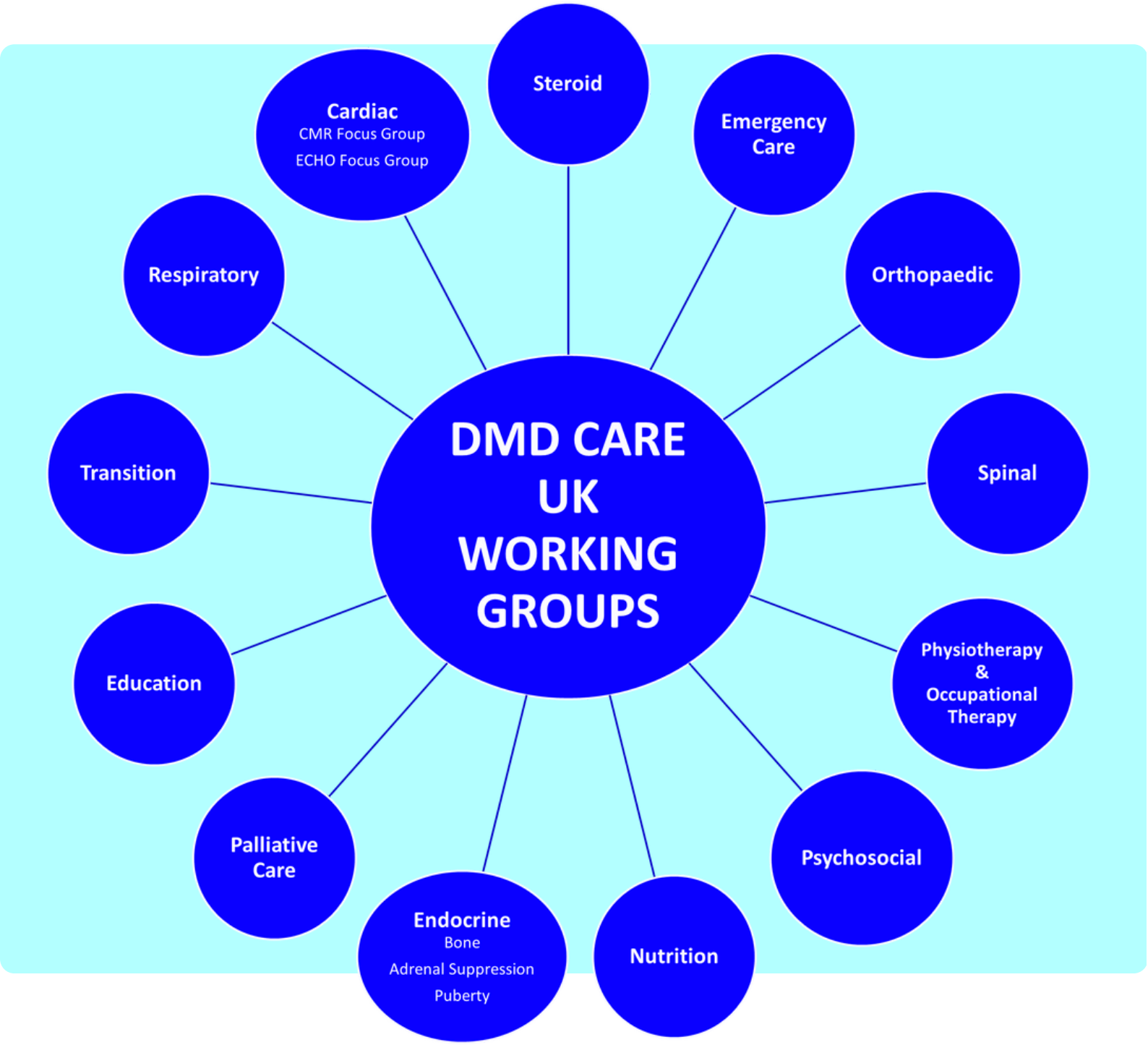
Steps to Standards of Care:

- Consult with multi-disciplinary clinical professionals via specialist working groups (WGs)
- Involve patients and patient experts
- Build consensus on guidance for that area of care
- Gain endorsement from the relevant national body
- Publish in peer-reviewed, open access journals
- Translate into patient- and family-friendly information materials
- Reach beyond DMD specialists
- Work with NICE and relevant professional bodies for guideline assessment and signposting

Working Groups

Our WGs are comprised of over 130 multi-disciplinary clinical professionals and more than 25 patient experts, offering their experience and knowledge.

This helps to build consensus on clinical recommendations for health care professionals as well as on patient and family care guidelines or which are used to develop patients and family guidelines.



27th & 28th March 2025

ANNUAL MEETING

This year's annual meeting brought together healthcare professionals, patient representatives and other stakeholders to discuss DMD Care UK's progress in the past year, ideas and future planning. It also provided learning opportunities about care standards through several workshop sessions.

Alex Johnson (right) opened Day 1. Alex is co-founder of Duchenne UK and Patient Lead for the DMD Care UK project This was held just before Duchenne UK's New Horizons conference, where up-to-date publications from our Working Groups were also presented directly to the patient community. The meting was organised by the DMD Care UK team and funded by Duchenne UK.





Professor Michela Guglieri presented a summary and next steps for the project at the end of the morning session.

Attendees

DAY 1

Thursday 27th March welcomed 50 in-person attendees including WG leads and patient experts.

Clinicians from additional North Star (NS) Network sites were invited to ensure all geographical areas were represented. The focus was on the progress of DMD Care UK over the past 12 months and agreement on priorities for the next year.

50

Morning attendees

80

Afternoon attendees

On Thursday afternoon a further 30 colleagues joined our workshop sessions on **psychological & psychiatric intervention and on corticosteroid treatment in DMD.** These included further HCPs from NS sites and patient experts.



Dr Rory Conn (project-funded psychiatrist) and Benjamin James (patient representative WG member) presented on how to assess and manage psychiatric presentations in DMD.

Attendees



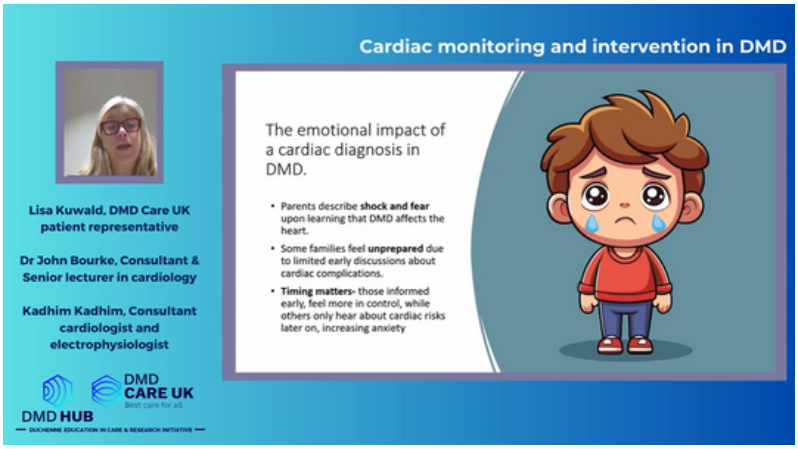
Benjamin James (patient expert and WG member) presented his own experiences of psychosocial support in DMD.

DAY 2

Educational workshops were delivered online and in person by clinical WG Leads and patient experts. Online sessions were recorded as part of the Duchenne Education and Research Initiative (DECRI).

Track 1	Track 2	Track 3
Cardiac monitoring & intervention **Online = 32** **In-person = 18**	**Long bone fractures** **Online = 29** **In-person = 12**	**Osteoporosis & vertebral fractures** **Online = 23** **In-person = 14**
Respiratory management in DMD **Online = 61** **In-person = 18**	**Managing the foot & ankle complex** **Online = 36** **In-person = 12**	**Delayed puberty in DMD** **Online = 19** **In-person = 14**

Lisa Kuhwald (patient expert and WG member) presented online to over 30 clinicians about the patient perspective of cardiac care in DMD.

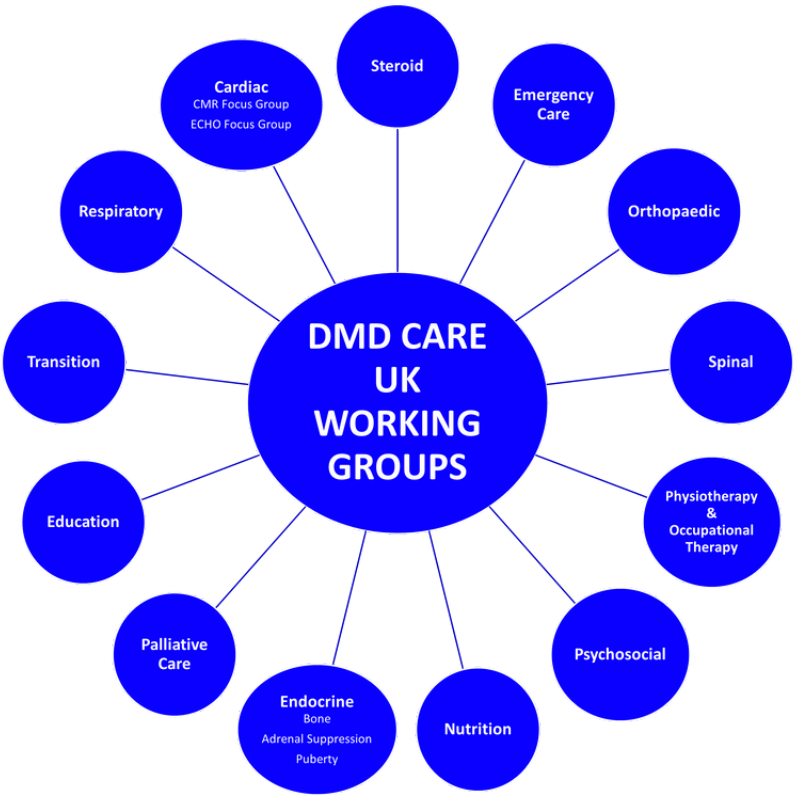


Session Summaries

DMD CARE UK PROJECT UPDATE, CATHY TURNER & PROF. MICHELA GUGLIERI

The meeting began with an overview of highlights from WGs. This then opened a discussion on additional areas for the project to prioritise in 2025-2026.

DECRI (the Duchenne Education in Care & Research Initiative) was introduced along with a variety of proposed research ideas.



AVERAGE RATING: 4.72/5 ★★★★★

FEEDBACK FROM ATTENDEES

- *I found this so useful - discussions around potential new areas of work was of particular interest.*
- *Fantastic start to the meeting and great to be thinking ahead to what gaps need addressing next.*
- *Superb session to begin.*
- *Amazing to hear all that you have achieved.*



Session Summaries

PSYCHOLOGICAL CARE & INTERVENTION IN DMD
DR CHLOE GEAGAN, DR LINDA BOUQUILLON & DR JANET HOSKIN

AVERAGE RATING: 4.74/5 ★★★★★

- *Always a complex and under resourced area. Recognition of common phenotypes interesting.*
- *[I] plan to explore use of Revised Children's Anxiety and Depression scale (RCADs) questionnaire in clinic.*
- *This was a really useful session with some good case studies to discuss. The group size led to some good interactive discussions.*
- *Really engaging and interactive sessions. A good explanation of the Bio-Psychosocial Model.*

HOW TO ASSESS AND MANAGE PSYCHIATRIC PRESENTATIONS IN DMD - WHEN THERE IS NO PSYCHIATRIST READILY AVAILABLE: A CASE STUDY APPROACH
DR RORY CONN & BENJAMIN JAMES

AVERAGE RATING: 4.85/5 ★★★★★

- *This was fantastic, amazing to discuss useful assessments to help with getting psychological support, neurodiversity etc. Also thanks so much to Benjamin for his first-hand experience!*
- *It was a great session to contribute to and share experience and expand on guidance and current WG thinking, progress and what we hope to achieve.*
- *Would be great to have more young men with Duchenne on WG groups.*
- *Useful discussion and opportunity for different opinions to be mentioned. Great that a lived experience was shared.*



Session Summaries

SIDE EFFECTS AND BENEFITS OF CORTICOSTEROIDS IN AMBULANT PATIENTS: PROF. MICHELA GUGLIERI & DR ANNE-MARIE CHILDS

AVERAGE RATING: 4.88/5 ★★★★★

- *Very interesting & good to discuss and hear the thoughts of others in the room.*
- *Good information comparing the three main steroids now available, with their side effects.*
- *Good information about the alert on adrenal suppression. I will look into getting this on our patient records.*
- *I found this the most helpful session of all: it included lots of discussion on vamorolone challenges*
- *Very useful going through the case studies & working examples.*

SIDE EFFECTS AND BENEFITS OF CORTICOSTEROIDS IN NON-AMBULANT PATIENTS: PROF. ROS QUINLIVAN & DR ANNA SARKOZY

AVERAGE RATING: 4.81/5 ★★★★★

- *Great session with good discussion - I think must have been very helpful for less experienced doctors and HCPs.*
- *Good to get useful information and to discuss new treatment options and their possible benefits versus side effects.*
- *I really liked the interactive side to these sessions about steroids.*
- *Very practical and helpful, thank you both.*

MANAGING ADRENAL INSUFFICIENCY AND THE RISK OF ADRENAL CRISIS: CORTICOSTEROID THERAPY DR JAROD WONG & DR HARSHINI KATUGAMPOLA

AVERAGE RATING: 4.76/5 ★★★★★



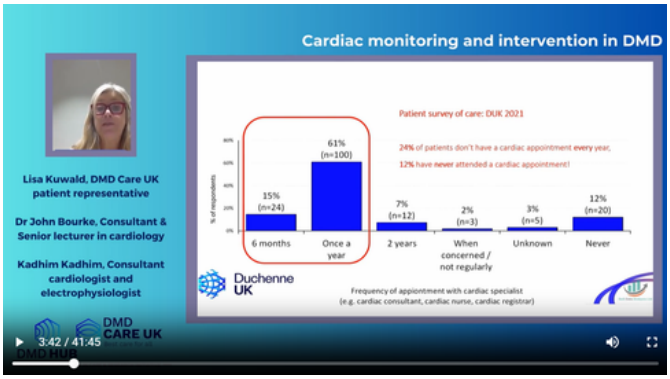
- *Very interactive session - really helpful for learning.*
- *Particularly [appreciated] information about vamorolone - equivalent dosing, stress dosing, interactions with other medication.*
- *Excellent session and gave a balanced view of vamorolone.*
- *Great comprehensive overview and how other centres flag their adrenal insufficiency patients.*

Session Summaries

TRACK 1

CARDIAC MONITORING & INTERVENTION: DR JOHN BOURKE, DR KADHIM KADHIM & LISA KUHWALD

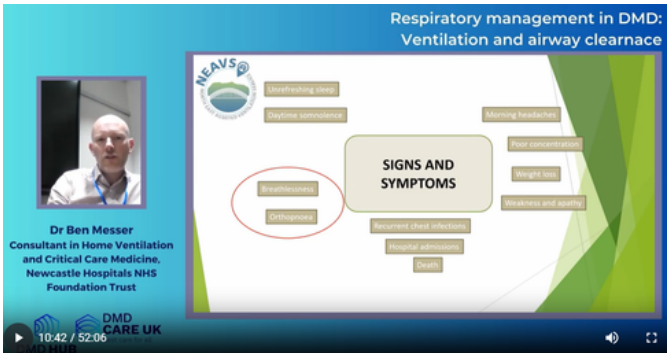
AVERAGE RATING: 4.83/5 ★★★★★



- *As always, these discussions teach me so much.*
- *Excellent talk giving real oversight.*
- *Very informative and will influence my management.*
- *Fantastic real-world advice on the challenges of managing young men with DMD.*
- *Our NM paediatrician is writing to the cardiac team to consider putting boys as young as 6 on heart meds after hearing [the] argument for starting treatment as soon as possible.*
- *The patient experience was interweaved with the clinical presentation - I thought this was done really well!*
- *Really illuminating discussions. Lots of new developments which we will need to reflect upon and see how it impacts on our practice.*

RESPIRATORY MANAGEMENT IN DMD: DR BEN MESSER

AVERAGE RATING: 4.92/5 ★★★★★



- *The best talk about ventilators I've ever heard*
- *Fantastic and in-depth session*
- *Very detailed explanation and well researched speaker. I learnt a lot.*
- *Really useful and interesting session.*
- *Very informative, well pitched.*
- *One of the best sessions I have attended. Clear and simple.*
- *Really enjoyed the session. Interesting insight on progression of respiratory failure & when to treat.*
- *Great, informative session with learning points that will impact my clinical practice.*
- *It was a very useful lecture/session. I wonder if the time slot could be extended even if by 30 mins to give time to think about the information.*
- *Brilliant session, very informative. Would really like to attend any follow up sessions.*

Session Summaries

TRACK 2

LONG BONE FRACTURES: DR MEREDITH JAMES, MR ROGER WALTON & KIRSTY OHLY

AVERAGE RATING: 4.74/5 ★★★★★



- *Ortho surgeon was very good, great to hear his proactive approach and understanding of DMD.*
- *Really knowledgeable speakers, the images were great to show the fracture management.*
- *It's great this is recorded so we can watch it back and share with colleagues - really helpful.*
- *Great to have x-rays and real-life examples to discuss. Enjoyed having input from both orthopaedic, physio and a mom to get all necessary viewpoints.*
- *This was extremely helpful! Very insightful session & I think lots of parents would benefit from a specific session on this.*
- *Nice discussion on the operative objectives and strategies in management of long bone fractures.*
- *Useful session, good to have the differing perspectives of orthopaedics, parents and physio.*

MANAGING THE FOOT AND ANKLE COMPLEX THROUGH THE LIFETIME OF SOMEONE WITH DMD | DR ANNA MAYHEW, MEL DIXON & DIONNE MOAT

AVERAGE RATING: 4.78/5 ★★★★★



- *Good useful tips & advice.*
- *Very good to have orthotist present.*
- *Great session. Would have liked more time for discussion but would have needed to be longer as wouldn't have wanted anything missed!*
- *Really useful discussion and reminder of the importance of getting the stretches done correctly by family/school and implementing orthotics at the right time.*
- *Excellent webinar - I found this so helpful and relevant, it really will help me in my clinical practice.*

Session Summaries

TRACK 3

DELAYED PUBERTY IN DMD & CONVERSATIONS ABOUT SEXUAL HEALTH & FERTILITY: DR CLAIRE WOOD & DR JANET HOSKIN

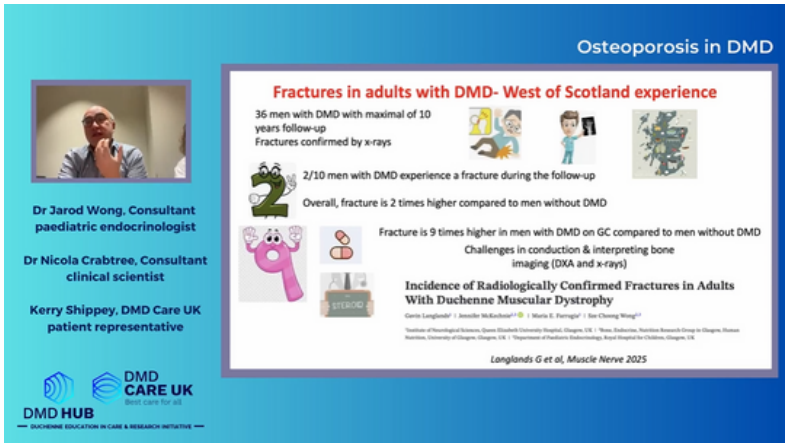
AVERAGE RATING: 4.91/5 ★★★★★



- *Fascinating session!*
- *Reinforced need to monitor for puberty delay. We will potentially explore referring all of our boys who are on long term steroids to endocrine team - if they will accept.*
- *Good useful tips & advice from this.*
- *Interesting, thought provoking and useful session. Thank you.*
- *Insight into some areas [I] have not thought about, [e.g. people with] DMD considering starting family.*
- *Very interesting to consider sexual education and difficulties in the context of DMD - difficult to discuss professionally and with patients, I think this is so important, thank you for discussing.*
- *I found the whole presentation really educational. I found the conversation around sexual health important as this is an area I would like to improve my knowledge in to help my patient group.*

OSTEOPOROSIS - HOW TO RECOGNISE A VERTEBRAL FRACTURE, MANAGING BISPHOSPHONATES SIDE EFFECTS & TRANSITION INTO ADULT CARE | DR JAROD WONG, DR NICOLA CRABTREE & KERRY SHIPPEY

AVERAGE RATING: 4.71/5 ★★★★★



- *Hadn't come across the issue of ongoing bisphosphonates following transition.*
- *I particularly enjoyed hearing [the parent] perspective.*
- *Excellent practical session and advice.*
- *Excellent talk - liked that it was based on a case study, also really enjoyed the radiology section - learnt a lot.*
- *Good insight into how some patients find it difficult to have DXA scan.*
- *Really good, Would be good to do a session focusing on [transition services] across the country.*
- *Very informative session.*

Impact

80+

In-person attendees

170+

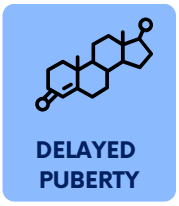
Dial-ins across the six online workshops

Increased reach through North Star Clinical Network, professional bodies and other networks.

















"We are improving care for people with DMD throughout the UK by developing practical guidelines, endorsed by professional bodies, published in open access journals and disseminated widely."

"I met with the paediatric team this week & there were some really healthy discussions on what they learned during the two days & how they will change practices going forward."

"Really illuminating discussions. Lots of new developments which we will need to reflect upon and see how it impacts on our practice. These discussions teach me so much, thank you."

Guidelines finalised then published → **Accompanying Patient & Family Guides** → **Scientific publication with endorsement**

NICE and DMD Care UK are working together to signpost and demonstrate robustness of DMD Care UK guidelines.

HCP Resources

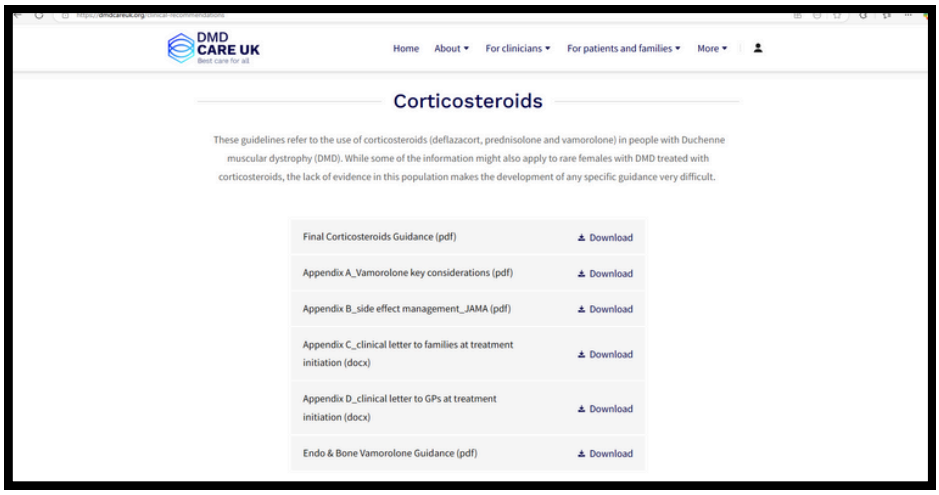
The Duchenne Education in Care and Research Initiative (DECRI) offers a training and education programme for **healthcare professionals (HCPs)** involved in clinical care and clinical trial delivery



https://decri-nmdtraining.talentlms.com/

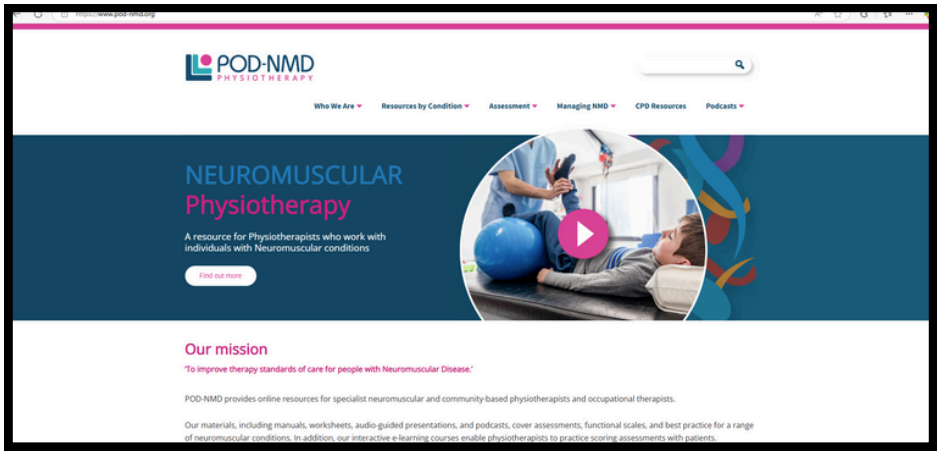
DMD Care UK website

https://dmdcareuk.org



POD-NMD provides online resources for specialist neuromuscular and community-based physiotherapists and occupational therapists.

https://www.pod-nmd.org/



Acknowledgements

DMD Care UK is a unique and powerful project aimed at delivering the best care for patients across the UK. Led by the JWMDRC at Newcastle and Duchenne UK, it is an equal collaboration between patients and clinicians, working together to create best-practice, robust guidance for all aspects of care that are easy to understand for clinicians and patients alike.

At the heart of the DMD Care UK collaboration, is a partnership that has been built over a decade between Duchenne UK and Newcastle. Over 13 years, Duchenne UK has invested over £3.1 million into the JWMDRC.

In 2016 Duchenne UK funded the **DMD Hub** – the creation of a network of clinical sites, with the main goal of ensuring all patients with DMD, both children and adults, have access to clinical research trials.

In 2020 Duchenne UK's funding enabled the launch of **DMD Care UK** which has now developed to include 13 working groups of multi-disciplinary clinical experts from across the North Star Network and patient representatives from around the UK.

Newcastle University

NHS
The Newcastle upon Tyne Hospitals
NHS Foundation Trust

THE John Walton
MUSCULAR DYSTROPHY
RESEARCH CENTRE

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Acknowledgements

Duchenne UK and the JWMDRC would like to take this opportunity to thank our incredible funders and collaborators: Sheli Rodney Director of Operations at the Duchenne Research Fund; Emma Hallam, Founder of Alex's Wish and Alex Johnson who is Founder of both Joining Jack and Duchenne UK.

Alex Johnson is also DMD Care UK's Patient Lead, and Duchenne UK have been instrumental in not only funding the project but in giving shared strategic insight, impact and guidance.

Professor Michela Guglieri is the Clinical Lead for the project, and the JWMDRC provide shared oversight of strategy, impact within the clinical community and project management across all 13 working groups.

Furthermore, we would not be able to run DMD Care UK without the expert input, insight and generous time from our clinician experts embedded in the North Star Clinical Network. They are critical for developing, publishing and disseminating the project's guidelines that have such an impact on the care of people living with DMD.